Universidad de Navarra

Facultad de Ciencias

MÁSTER EN INVESTIGACIÓN BIOMÉDICA

Research Project Proposal

Academic year 2025-2026

Project Nº 14	
Title: *Optimization of AAV renal delivery tin pigs and NHPs*	
Department/ Laboratory Gene therapy for rare diseases program, CIMA	
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Summary

Chronic kidney disease (CKD) is estimated to affect a 13% of the population worldwide and is a leading cause of global mortality1. Up to a 30% of CKD cases are attributed to inherited kidney diseases (IKDs) caused by the malfunction of a single gene1. Autosomal dominant polycystic kidney disease (ADPKD) is the most frequent IDK, with a frequency of 1:1,000. ADPKD is characterized by the appearance and expansion of multiple cysts in the kidney that lead to progressive loss of kidney function and decrease of glomerular filtration rate. Commonly, patients develop end-stage renal disease (ESRD). The current treatments aim to reduce the progression of kidney damage by regulation of diet and water intake, as well as management of hypertension, a common side effect of the decline of kidney function. Mutations in PKD1 and PKD2 genes account for most of the cases of ADPKD, 78% and 15% respectively. The only curative treatment for these diseases is kidney transplantation. These diseases can benefit from gene therapy approaches. Among the most popular and safe vector are those based on Adenoassociated Virus (AAVs). They have been shown to target different organs, such as peripheral nervous system, retina and liver. However, despite their promise, AAVs still show some limitations, including the restricted transduction of certain organs, such as kidney. In this work we will generate libraries of AAV which transduction will be tested in the kidney in pigs and NHPs, Furthermore, we will develop a medical device for an efficient and safe administration of the AAV vectors, that will be evaluated in large animals.

yes	X
no	

Does the project include the possibility of supervised animal manipulation to complete the training for animal manipulator?